## Phenylketonuria (PKU) (mRNA-3283)

*Last program update: October 29, 2020*

<table>
<thead>
<tr>
<th>Modality</th>
<th>ID #</th>
<th>Program Indication</th>
<th>Preclinical development</th>
<th>Phase 1</th>
<th>Phase 2</th>
<th>Phase 3 and commercial</th>
<th>Moderna rights</th>
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<td>Systemic Intracellular therapeutics</td>
<td>mRNA-3927</td>
<td>PCCA/PCCB; Propionic acidemia, PA</td>
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<tr>
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<td>mRNA-3705</td>
<td>MUT; Methylmalonic acidemia, MMA</td>
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<td>mRNA-3283</td>
<td>PAH; Phenylketonuria, PKU</td>
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*mRNA-3283 is in IND-enabling GLP toxicology studies*
Phenylketonuria (PKU) overview

Opportunity to address unmet need

- PKU is a rare inherited metabolic disease resulting from a deficiency in the metabolism of phenylalanine, or PHE, due to mutations within the enzyme phenylalanine hydroxylase, PAH.

- **Disease burden:** Progressive mental disability from tyrosine deprivation.

- **Target population:** Incidence of ~1:10K-15K live births in the US
  —~21,000-32,000 patients in the US

- Approved therapies include:
  —20-56% of patients respond favorably to BioMarin’s Kuvan
  —Non responsive patients treated with restricted diet
  —Biomarin’s Palynziq was approved in May 2018

Moderna concept: IV-administered mRNA encoding PAH enzyme to restore deficient or defective intracellular enzyme activity.
Phenylketonuria (PKU) (mRNA-3283)

Preclinical data – restoration of enzymatic activity

Study Design:

- **Species:** Mouse
- **Animals:** PAH-/
- **Dose:** 0.5 mpk
- **Dosing Schedule:** weekly
- **Injection Route:** IV
- **Sample Size:** [5-6]

**PHE reduction with repeat dosing of PAH mRNA**

*Data not collected due to a snow storm*
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